Biotechnology and Public Health: Scenarios to 2030

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International Futures Project:
Bioeconomy to 2030: Designing a Policy Agenda

OECD activities in biotechnology

- Provides forum for discussion, policy development, statistics and analysis, evaluation of future trends.
- Many directorates and divisions involved: Environment, Agriculture, Biotechnology Division, Transport, International Futures.
- Both guidelines and reports adopted by the OECD council *and* working documents etc. with no official status.

Bioeconomy to 2030 project

- Trends to 2015 on the health, industry and agricultural applications of biotechnology
- Scenarios to 2030
- Business model analysis
  - Technological developments
  - Role of publicly financed research sector
  - Regulatory policies
  - Market competition, rise of Asia
- Policy recommendations

OECD Policy priorities

- Improve efficacy (health benefits) and efficiency (lower costs) of innovation.
- Reduce development times for NCEs, therapies, etc.
- More evidence based medicine – including for biological markers.
- Develop regulatory environment for access, use and linkages of public and private data sets, from risk factors (genetics) to outcomes (prescribing & health).
- Encourage preventive and personalized health care.

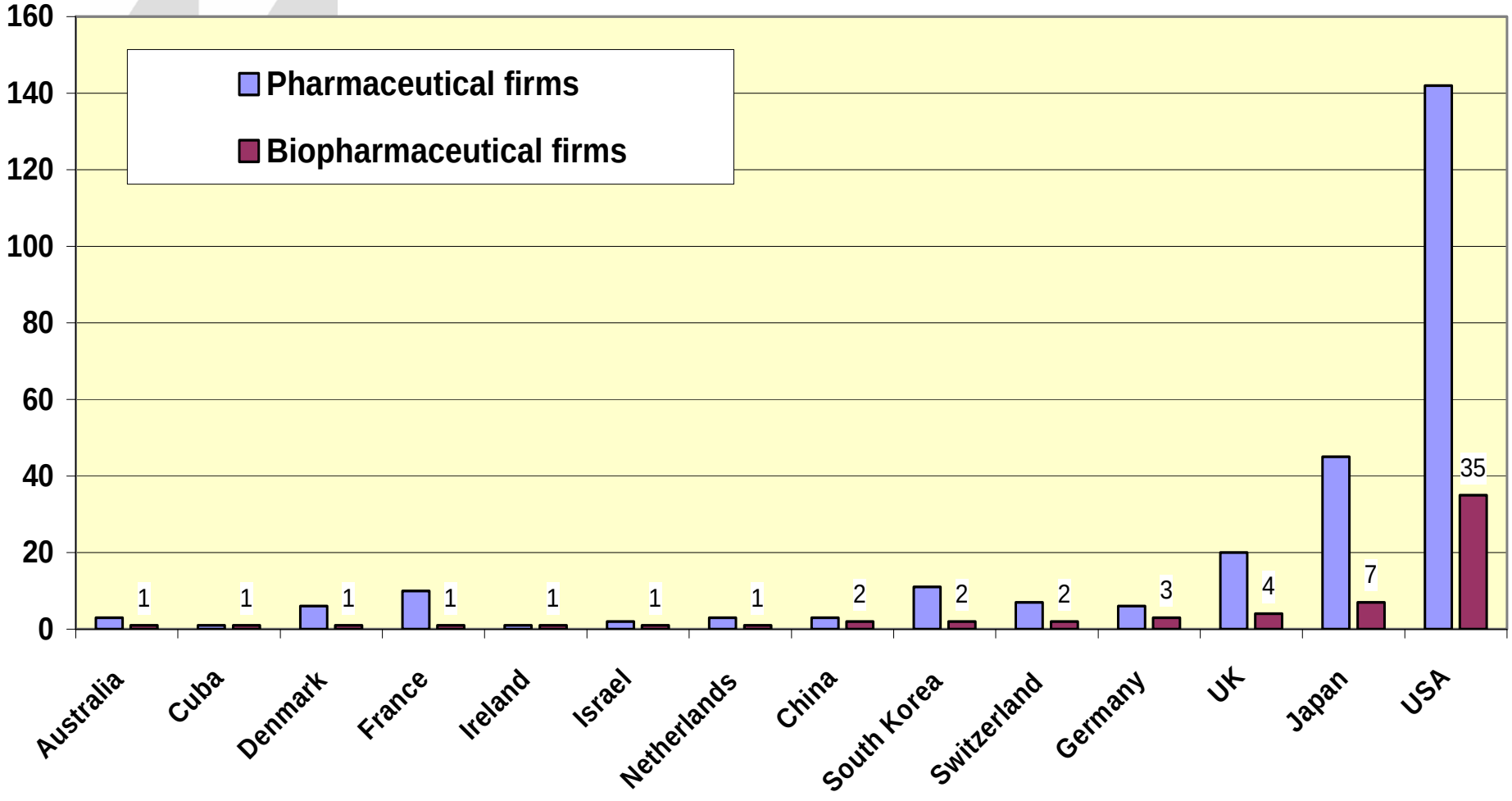
Trends to 2015

Trends in Health Biotechnology

- **Problems:**
  - What is biotechnology?
  - Statistics and indicator availability
    - Data for large molecule biopharmaceuticals, vaccines & invasive diagnostics
    - No data for many other applications, such as the use of biotechnological knowledge to develop small molecule pharmaceuticals

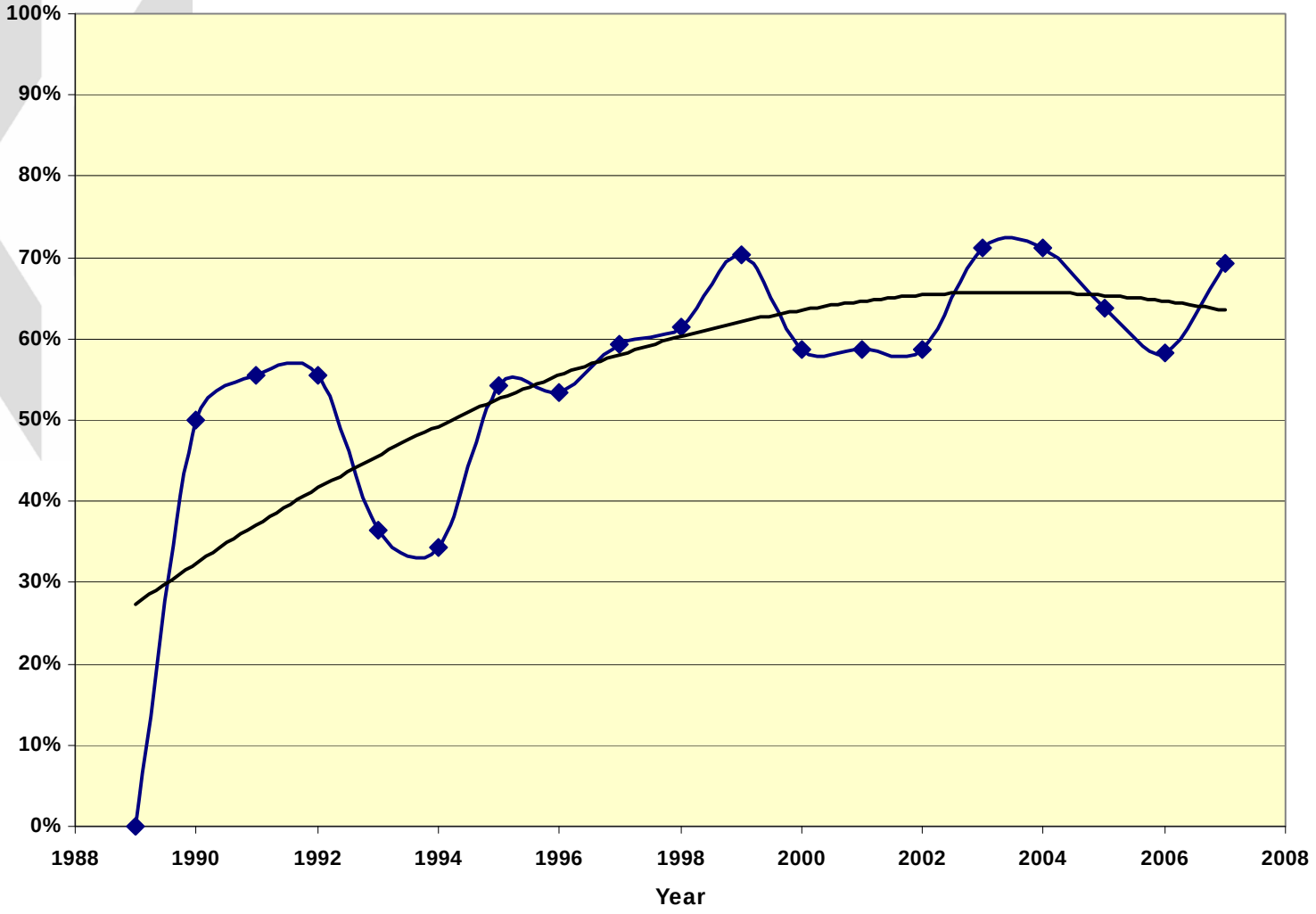
Pharma and biopharma firms, by country

At least one NCE on the market



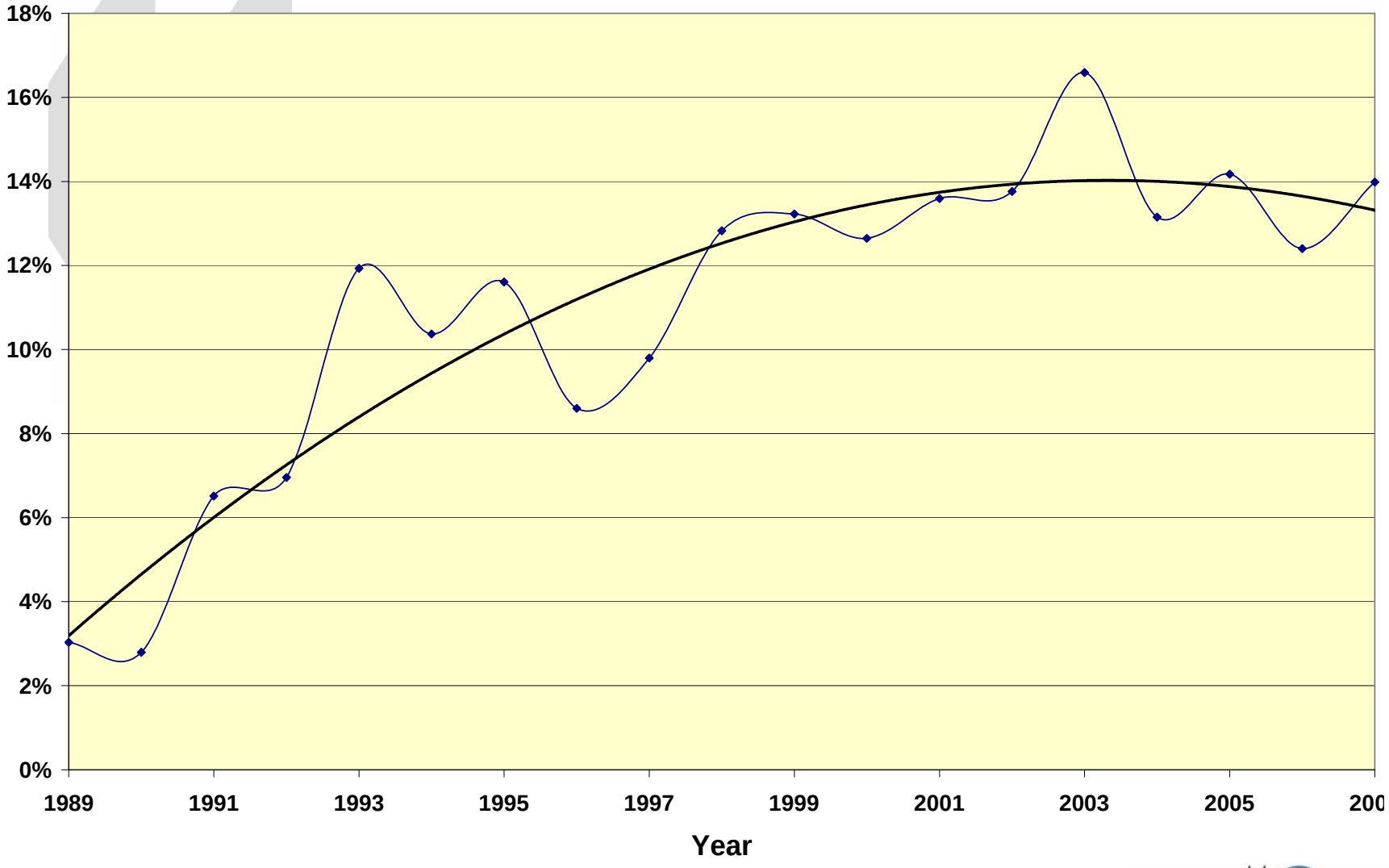
Source: OECD, based on data from PHARMAPROJECTS.

US share of all biopharmaceuticals



Source: OECD, based on data from PHARMAPROJECTS.

Biopharmaceutical products as a share of all pharmaceuticals (3-year running average)



Source: OECD, based on data from PHARMAPROJECTS.

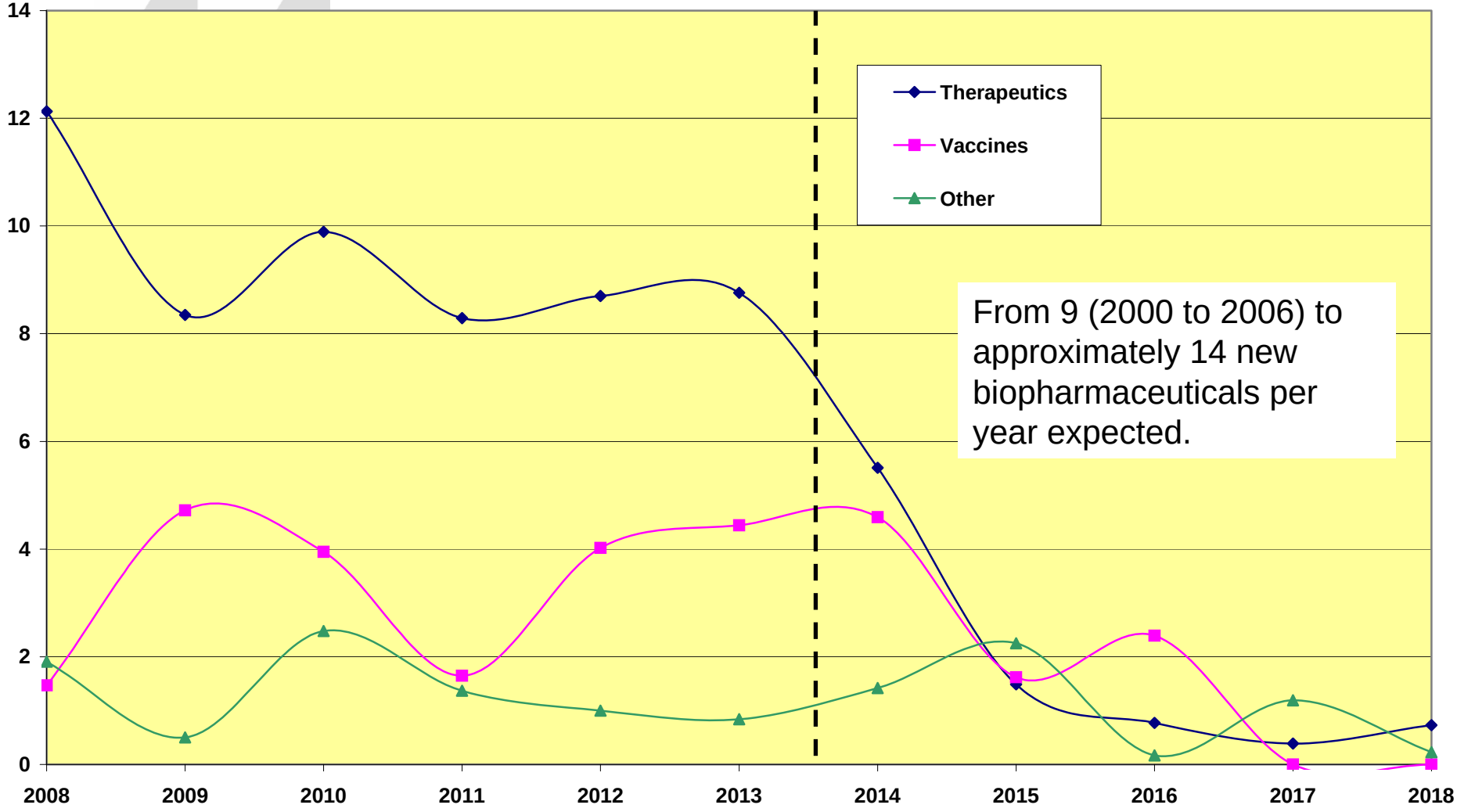
Types of bio-NMEs currently in clinical trials

	Phase 1	Phase 2	Phase 3	Pre-registration	Total
Monoclonal Antibodies	63	54	22	3	142
Recombinant vaccines	49	57	7	1	114
Recombinant therapeutics	18	45	11	7	81
Other	27	38	8	0	74
Gene therapy	12	43	7	2	64
Stem and other cellular therapy	18	33	9	2	62
Antisense therapy	8	24	0	1	33
Total	195	294	64	16	570

Source: OECD, based on data from PHARMAPROJECTS.

- Research on experimental therapies (in blue) is largely (92.7%) undertaken by small DBFs.
- Conflicts with Pisano's (2006) recommendation that highly novel drug development works better in fully integrated firms.

Bio-NME products expected to reach registration, by year



Source: OECD, based on data from PHARMAPREDICT.

Products estimated to reach the market, by phase

	Biotechnology		Other Pharmaceuticals		Biotech Share
	Total Trials	# est. to reach market	Total Trials	# est. to reach market	Biotech % of all drugs
Preclinical	942	47.1	3432	250.22	18.82%
Phase I	213	44.73	917	290.72	15.39%
Phase II	310	96.1	1206	509.61	18.86%
Phase III	73	45.99	324	232.72	19.76%
Pre-registration	18	15.84	86	76.51	20.70%
Total	1556	249.76	5965	1359.78	18.37%

Source: OECD, based on data from PHARMAPREDICT.

Other estimates of a biotech share of 30% to 50% use a different definition of health biotechnology.

The black hole for statistics

- Use of biotechnological knowledge to develop new **small molecule pharmaceuticals**:
  - Target identification
  - Pharmacogenetics / genomics
  - Systems Biology

The biotechnology advantage

	Biopharmaceuticals (all indications)		All other drugs (all indications)	
	N	%	N	%
Major, important, or some advance	29	24.3%	248	13.8%
Minimal advance	40	33.6%	424	23.7%
No advance (me too)	27	21.8%	899	50.2%
Not acceptable	13	10.9%	115	6.4%
Judgment reserved	11	9.2%	104	5.8%
Total	*119*	*100%*	*1,790*	*100%*

Source: OECD, based on data from *PRESCRIRE*

- Biotechnology, so far, has offered greater therapeutic advances than other drugs – new modes of action.
- Therapeutic advance may be declining over time, but this trend could be reversed by experimental treatments in the pipeline.

Therapeutic value by firm size

		Therapeutic advance over previous treatments			
Firm employees	**Number of biopharmaceuticals**	**Important or some advance**	**Minimal or no advance**	**Not acceptable**	
< 10,000	30	43.3%	**36.7%**	20.0%	100.0%
10,0000+	35	25.7%	**68.6%**	5.7%	100.0%
Total	65	33.8%	53.8%	12.3%	100.0%

Source: OECD, based on UNU MERIT database for 65 biopharmaceuticals (excluding vaccines and diagnostics) that have been assessed by *Prescrire*

Diagnostics

- Over 1400 gene-based tests for diseases:
  - Not sure how many are clinically informative – availability by country varies from 214 in Spain to 751 in US.
- Tests for multi-gene risk factors for diabetes.
- In vitro diagnostics (IVD) using biotechnology (immunoassays and nucleic acid tests)
  - Accounted for an estimated 30% of global IVD market in 2004.
- By 2015, expect multi-gene testing for susceptibility to many diseases to be common.
- Increasing use of diagnostics linked to prescribing practices.

Bioinformatics 1

- Predictive medicine: genetic testing for risk factors.
- Pharmacogenetics, etc: improved targeting of pharmaceuticals (HerceptTest), response to other therapies.
- Should both be increasingly common by 2015.
  - Will partly depend on net costs versus benefits.
  - Genetic testing uptake requires protocols, standards and validation.

View of Munich Re

- Monogenetic disorders (cystic fibrosis, Duchenne's, Huntington's) account for approximately 1% of the potential for genetic testing.
- Multi-gene testing for risk factors for complex diseases (cardiovascular, diabetes, cancer, neurological etc) account for the other 99%.
  - Multi-gene testing will take off after 2012, as costs fall.
- Why does an insurance firm care?
  - Effects of asymmetric knowledge on health coverage.
  - Impacts on health care costs.

Bioinformatics 2

- Large scale population-based databases of health outcomes, prescriptions, treatments.
  - Hall and Lucke (2007): impact of prescriptions on health outcomes.
- Post market follow-up: substantially better data on interactions, adverse effects, etc.
- Already feasible in some jurisdictions, but still serious limits due to confidentiality.

What do trends to 2015 tell us?

- Biotechnology based therapies will play a minor although increasing role in health care up to 2015.
- New therapies based on antisense, stem cells, and gene therapy are unlikely to be in wide use.
- Gradual development of diagnostic and pharmacogenetic technologies that could form the foundation of larger scale changes to health care.

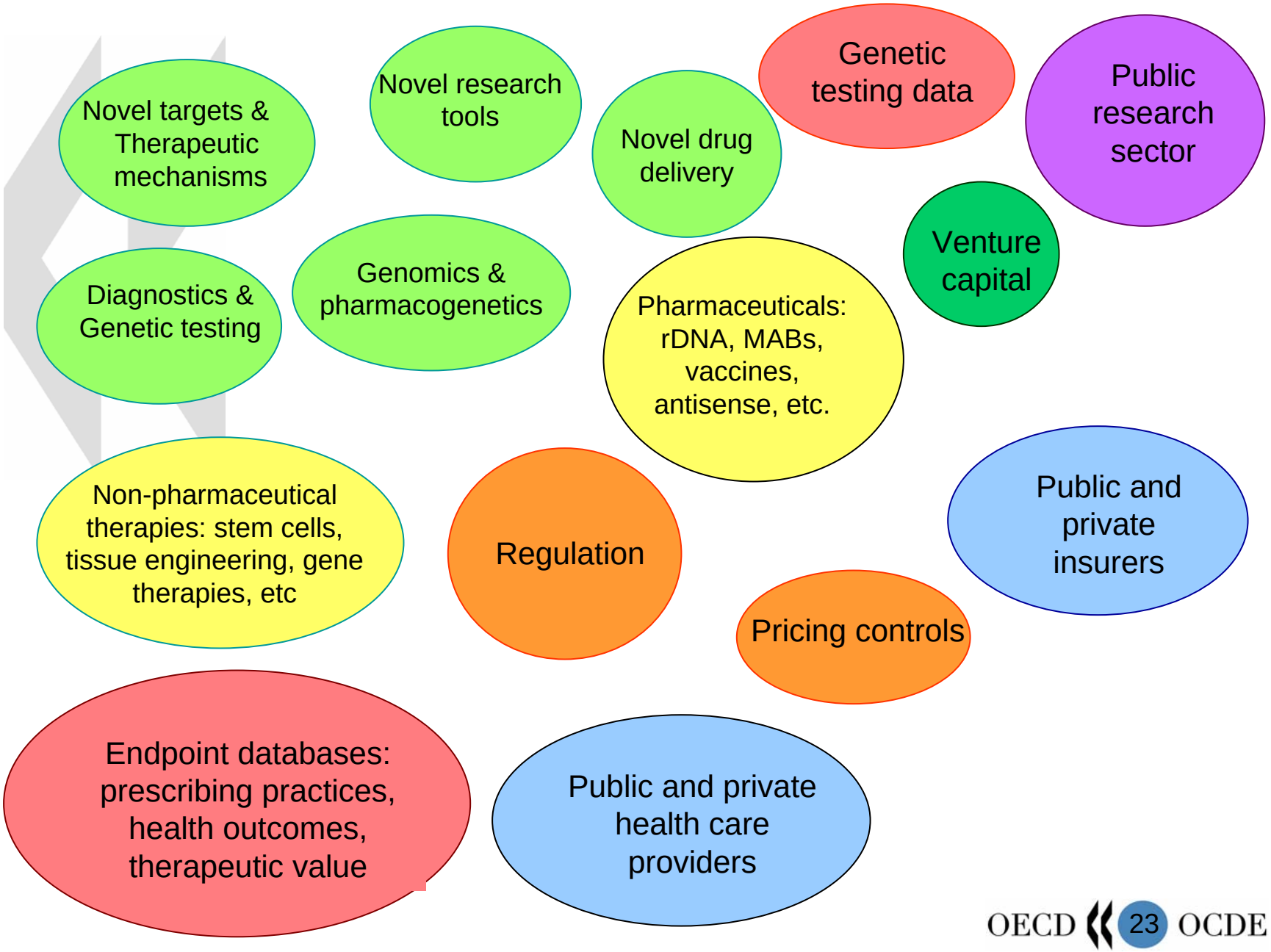
Transition phase from current health care system to a future 'biotechnology' system.

Health Scenarios to 2030

Source: Joyce Tait

Purpose

- Think through implications of technological developments on society, economics (costs), innovation strategies, etc.
- Not necessary to guess correctly – simply to think through ‘what if’ policy implications.
- Doesn’t take much to see potential – problem is finding a solution to how to get there (transition economics).
- Scenarios help with thinking about this.


Novel targets & Therapeutic mechanisms
Novel research tools
Novel drug delivery
Genetic testing data
Public research sector
Venture capital
Diagnostics & Genetic testing
Genomics & pharmacogenetics
Pharmaceuticals: rDNA, MABs, vaccines, antisense, etc.
Non-pharmaceutical therapies: stem cells, tissue engineering, gene therapies, etc
Regulation
Public and private insurers
Pricing controls
Endpoint databases: prescribing practices, health outcomes, therapeutic value
Public and private health care providers

Technical scenario

- Substantially greater focus on prevention and risk management, due to genetic testing; combined with personalized medicine.
- Integration of genetics *and* post marketing information in both drug regulation and in 'fine tuning' treatment therapies.
- Stem cells – cures rather than treatments reduce markets for block buster drugs.
- Fragmented markets due to pharmacogenetics, gene testing for risk factors, greater use of preventive health care due to identification of risks.

Social scenario

- Testing to identify genetic risk factors inexpensive and common by 2015, but people slow to adopt preventive strategies – diet (neutraceuticals?), exercise, etc.
- Health effects of the obesity pandemic (plus end of benefits from lower smoking rates) causes the past increase in the average lifespan of 2.5 years per decade to cease around 2015.
- Rapidly rising health care costs, in part from new technologies, combined with little improvement in health, increases resistance by 2020 to higher health care costs – more difficult for firms to recoup high costs of investment in R&D.
- “Avastin” model of improved health care technology, or stem cell breakthroughs and cures?

Economic scenario

- Can we get past, in time, a period of increasing health care costs with little benefit?
  - Or will both investment and willingness-to-pay dry up first?
- Insurer view: people will pay for increased health care costs if there is a large benefit, but will resist increased costs with little benefit.
- What is required to make this transition?

Health scenario - integration

- Tait (2007): 'Networked Health Care' - Integration from drug discovery through to health care provision, based on an 'ICT" information network.
  - New business model based on a joint venture by a major ICT and major pharmaceutical firm.
  - Does not require a blockbuster model – package of products sourced from a variety of firms.
  - Coordinate public and private sector providers of drugs, other treatments, and services.
- Focus on reducing health care *system* costs.

- Pisano (2006): Improved integration for drug development to overcome problems of information asymmetry, specialised assets, tacit knowledge, and IP uncertainty.
  - Return of ‘large pharma’
  - Improvements in translational medicine, more sophisticated patenting policies by universities.
- Focus on reducing *innovation* costs.

Integration as the solution?

- Tait: A main problem is the regulatory system, which creates barriers to entry for small firms and stifles innovation.
  - Integrated systems that combine data from personal genetic testing, pharmacogenetics, and large health outcome databases?
  - End of clinical trials as we know them today?
- Pisano: Regulation is not the main problem, with barriers due to portfolio economics (a large number of projects is needed for a successful ‘hit’) and problems in improving the efficiency of innovation.

How do we get there?

- Integration will be essential and probably include both the Pisano and Tait conceptions.
- Regulation – can current systems be tweaked to both enable innovation and ensure substantial improvements in efficacy of new therapies?
- How do we pay for health care innovation?
- What new business models will be required to both support innovation and provide a ‘payer’?